American Center for CURES

Addressing Findings of the *National Academy of Sciences* Report: "Enhancing the Vitality of the National Institutes of Health: Organizational Change to Meet New Challenges"

Background

The health challenges facing the US and the world today are a mix of infectious diseases, such as HIV, tuberculosis and malaria, long-standing chronic such as diabetes and cancer, and new emerging threats, such as SARS and avian influenza. In the context of these growing concerns, Congress commissioned the National Academy of Sciences (NAS) in 2001 to report on "whether the current structure and organization of NIH are optimally configured for the scientific needs of the twenty-first century." Indeed, NIH is America's premier public research investment and between 1998 and 2003, the NIH budget of $14 billion dollars doubled to $28 billion. By commissioning the NAS report, Congress wanted to know how it might better influence its burgeoning research investment. Congress solidified its support for the NIH but simultaneously posed questions of vitality for the NIH to address domestic and global health needs:

- Are the 27 NIH Institutes and Centers able to coordinate their research goals and priorities to reflect the multidisciplinary nature of today's health problems?
- How is the NIH producing and sharing biomedical knowledge from multiple disciplines to spur the development of clinical tools, drugs, and other therapies to battle longstanding and emerging diseases?
- Can the NIH respond effectively to acute health threats, such as to burgeoning HIV infection rates and the threat of a bioterrorism attack?
- Is the NIH cultivating the next generation of researchers to build upon the great works of NIH past?

The end result was the 2003 NAS report, "Enhancing the Vitality of the National Institutes of Health: Organizational Change to Meet New Challenges". The report reinforced NIH successes over the last 50 years as the national and global leader in biomedical research. NIH accomplished this by developing a cutting edge internal research infrastructure and a democratic extramural grant program that almost single-handedly supports University-based research in the biological sciences. However, the report also cautioned that "no organization as important as NIH should remain frozen in organizational space" and any changes in organizational structure to achieve greater progress in chronic and emerging diseases, however essential, would be fraught with difficulty and risk.

NAS Report Findings

The NAS report made a total of 14 recommendations[1]. In the final analysis, the NAS report recommended maintaining the general structure of NIH to ensure NIH's strengths would be protected: conducting essential basic science, and disease, behavioral, organ, and system based research in its intramural program and funding peer-reviewed grants to University researches in its extramural program. However, the report also recognized the need for organizational changes which could help Institutes work across their respective stovepipes, foster a culture of risk-taking and innovation, and give the NIH director, other leadership, and the public the power to prioritize NIH research to solve the nation's most burdensome health problems. Collectively, these changes would enhance the capacity of the NIH to not only pursue fundamental knowledge about the nature and behavior of living systems, *but to apply that knowledge to extend healthy life and reduce the burdens of illness and disability*. This is NIH's mission.

CURES Addresses the 6 Key Recommendations of the NAS Report:

1. Strengthen Clinical Research: The NAS report recommended that the NIH "pursue a new organizational strategy to better integrate leadership, funding, and management of its clinical research enterprise". Senators Lieberman and Cochran's Cures proposal creates the American Center for Cures (ACC), headed by a Cures Director. One of the new Director's key charges will be to promote and simplify the clinical research endeavor. The Director will establish a national electronic registry and results database for clinical trials in order to increase enrollment of research subjects and improve sharing of efficacy and safety outcomes emanating from the clinical research endeavor. The Director will fund multidisciplinary clinical research teams in the academic and private sector, create Centralized Internal Review Boards (CIRB) to simplify the human subjects review processes for multi-institutional clinical trials, and augment NIH investments in training the clinical research workforce of the future.

2. Enhance and Increase Trans-NIH Strategic Planning and Funding: The 27 NIH Centers and Institutes with their own directors and budgets generally operate independently. The resulting structural and organizational stovepipes cannot capitalize on the NIH's collective research capacity to address complex problems from different fields. For example, the problem of diabetic retinopathy should be tackled by researchers in the Institutes of the Eye, Diabetes, Digestive and Kidney disease, Biomedical Imaging and Bioengineering, and Allergy Immunology and Infectious disease. To address this problem, Cures puts forth new and significant funding for innovative multidisciplinary collaborative research across NIH institutes and centers. NIH Insititute and Center

[1] See appendix

Directors on the Cures Council will be entrusted to coordinate the intramural research agenda with that of the ACC. A new Office of Intramural Risk Opportunity and Mapping will oversee the intramural research programs of the NIH to be certain they are complementary, non-duplicative, and distinct from extramural and private programs.

3. Strengthen the Office of the NIH Director: The NAS report emphasizes the need for the NIH Director to have more budgetary support and flexibility. The current NIH Director, Dr. Zerhouni, has taken these steps with the NIH Roadmap. The Cures legislation further supports the spirit of the NIH Roadmap with organizational and funding commitments that bring the translational research investment to necessary and appropriate scale. The NIH Director will recommend appointees for the Cures Director to the President. The NIH Director will be a co-chair of the ACC Council that will set the research and funding priorities for translational research projects at the NIH. The NIH Director will head efforts to establish a public electronic database for clinical trials and NIH funded publications. The NIH Director will establish translational research training programs within each NIH Institute and Center, among other initiatives.

4. Create a Director's Special Projects Program: The NAS committee recommended the creation of a program to support strategic risk taking and high-potential payoff research. The Department of Defense has had significant success with its Defense Advanced Research Program Agency, where a group of expert portfolio managers invest in and oversee innovative, multidisciplinary, collaborative projects to advance specific fields or to develop needed technologies. A Health Advanced Research Program Agency (HARPA) will be established within the ACC to help lead breakthrough advances in biomedical research, such as a vaccine against HIV or genetic probes pivotal to the elucidation of disease producing genes. HARPA will have the authority to flexibly fund project, including the prompt awarding, releasing, enhancing and withdrawal of monies.

5. Promote Innovation and Risk-Taking in Intramural Research: The NAS report recommended that the NIH intramural research portfolio be distinct from that of the extramural program and private sector. This obligates the intramural program to take risks and to be innovative. The Office of Intramural Risk Mapping established within the ACC will identify and map health risks and scientific opportunities for the NIH's intramural program. It will share this information with the ACC Advisory Council that helps the Cures Director set ACC research priorities. HARPA funds will further encourage collaboration and "out of the box thinking" when it come to research amongst the NIH Institutes and Centers.

6. Standardize Data and Information Management Systems: The NAS committee recommended that the NIH must increase its capacity for data gathering and reporting to meet its obligations "…for effective management, accountability, and transparency". Cures seeks to improve the sharing of information by providing funding to the National Library of Medicine (NLM) to create and maintain a publicly accessible database of all

publications resulting from NIH-funded research. It establishes a national electronic registry and results database to increase enrollment in public and private clinical trials and to share efficacy and safety outcomes emanating from the clinical research endeavor. In addition, Cures focuses on the need to grow the NLM facilities according to the demands of new scientific discoveries and fields, especially within the areas of genomics and proteinomics.

CURES versus the NIH Roadmap

In response to the NAS report, NIH Director Dr. Elias Zerhouni launched the NIH Roadmap in FY 2004 with $128 million in funding from existing NIH budget allocations. Funding increases every year until FY 2009 and tops out at $507 million. The NIH Roadmap consists of:

- **New Pathways to Discovery** to obtain a deeper understanding of biological systems based on new models
- **Research Teams of the Future** to facilitate collaboration across institutes by awarding grants to support institutional partnerships and cutting-edge research
- **Re-engineering the Clinical Research Enterprise** reforms the clinical trial process to allow for broader participation from community-level patients and providers

While the NIH roadmap addresses some of the concerns of the NAS report, it does not address key provisions including increasing the power of the NIH Director, establishing an advanced research projects agency, and establishing a new leadership position that can facilitate the clinical research essential to moving products faster from bench to bedside. Unlike CURES, the roadmap relies on traditional academic-government relationships. *CURES cultivates new relationships between NIH researchers and innovative industrial partners.* Unlike the roadmap, which asks the NIH to focus on new priorities with old tools and funds, *Cures provides much higher levels of funding for a Center uniquely devoted to translating research to produce new diagnostics, therapies and even cures to the most important diseases.*.

Appendix

Recommendations from the NAS report, "Enhancing the Vitality of the National Institutes of Health: Organizational Change to Meet New Challenges":

Recommendation #1: Centralization of Management Functions

Recommendation #2: Public Process for Considering Proposed Changes in the Number of NIH Institutes and Centers

Recommendation #3: Strengthen Clinical Research

Recommendation #4: Enhance and Increase Trans-NIH Strategic Planning and Funding

Recommendation #5: Strengthen the Office of the NIH Director

Recommendation #6: Establish a Process for Creating New OD Offices and Programs

Recommendation #7: Create a Director's Special Projects Program

Recommendation #8: Promote Innovation and Risk Taking in Intramural Research

Recommendation #9: Standardize Data and Information Management Systems

Recommendation #10: Set Terms and Conditions for IC Director Appointments and Improve IC Director Review Process

Recommendation #11: Set Terms and Conditions for the NIH Director Appointment

Recommendation #12: Reconsider the Status of the National Cancer Institute

Recommendation #13: Retain Integrity in Appointments to Advisory Councils and Reform Advisory Council Activity and Membership Criteria

Recommendation #14: Increase Funding for Research Management and Support